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For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: A SCREENING METHOD AND COMPOUNDS FOR TREATING FRIEDREICH ATAXIA

(57) Abstract: The present invention relates to a method for identifying and/or validating candidate substances for the treatment of Friedreich Ataxia (FRDA). Furthermore, the present invention relates to the use of selenium, Ebselen and Glutathione peroxidase (GPX) mimetics for the preparation of a medicament for the treatment of FRDA. Another aspect of the present invention relates to the use of cells with reduced frataxin expression for identifying and/or validating candidate substances for the treatment of Friedreich Ataxia.



## INTENATIONAL SEARCH REPORT

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Documentati	ion searched other than minimum documentation to the extent that sur	ch documents are included in the fields se	arched	
	ata base consulted during the International search (name of data base ternal, BIOSIS, MEDLINE, WPI Data, PA		)	
C. DOCUME	ENTS CONSIDERED TO BE RELEVANT			
Category °	Citation of document, with indication, where appropriate, of the rele	vant passages	Relevant to claim No.	
х	FRYER M J: "Rationale for clinic of selenium as an antioxidant for treatment of the cardiomyopathy o	the	8,9	
	Friedreich's ataxia." MEDICAL HYPOTHESES, vol. 58, no. 2, February 2002 (20			
Y	pages 127-132, XP001095763 ISSN: 0306-9877 abstract; table 1		21,22	
			-	
X	PILCH JACEK ET AL: "Friedreich's JOURNAL OF CHILD NEUROLOGY, vol. 17, no. 5, May 2002 (2002-05 315-319, XP001095762 ISSN: 0883-0738	8,9		
A	page 318, column 1, paragraphs 2-	4	1-7, 10-26	
		/		
X Fun	ther documents are listed in the continuation of box C.	χ Patent family members are listed	l in annex.	
° Special ca	ategories of cited documents:	T' later document published after the intermediate and a second control of the co	ernational filing date	
consi	ent defining the general state of the art which is not dered to be of particular relevance document but published on or after the international	or priority date and not in conflict with cited to understand the principle or the invention	eory underlying the	
filing filing	date ent which may throw doubts on priority claim(s) or	<ul> <li>"X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone</li> <li>"Y" document of particular relevance; the claimed invention</li> </ul>		
citatio	on or other special reason (as specified) nent referring to an oral disclosure, use, exhibition or means	cannot be considered to involve an it document is combined with one or m ments, such combination being obvious	iventive step when the ore other such docu-	
'P' docum	ent published prior to the international filling date but	in the art. "&" document member of the same paten		
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### INT NATIONAL SEARCH REPORT

Internation No PCT/EP 03/07006

		PC1/EP 03/0/006
	ation) DOCUMENTS CONSIDERED TO BE RELEVANT	
Category °	Citation of document, with Indication, where appropriate, of the relevant passages	Relevant to claim No.
Y	LODI R ET AL: "Mitochondrial dysfunction in Friedreich's ataxia: from pathogenesis to treatment perspectives." FREE RADICAL RESEARCH. SWITZERLAND APR 2002, vol. 36, no. 4, April 2002 (2002-04), pages 461-466, XP001097344 ISSN: 1071-5762	21,22
A	page 464, column 1, paragraph 2 —column 2, paragraph 2	1-20, 23-26
Υ	US 6 133 322 A (RUSTIN PIERRE ET AL) 17 October 2000 (2000-10-17)	21,22
A	column 4, line 24 —column 7, line 18; claims 1—18; example 3	1-20, 23-26
Υ	RUSTIN PIERRE ET AL: "Heart hypertrophy and function are improved by idebenone in Friedreich's ataxia." FREE RADICAL RESEARCH. SWITZERLAND APR 2002, vol. 36, no. 4, April 2002 (2002-04), pages 467-469, XP001097343 ISSN: 1071-5762 abstract	21,22
<b>Y</b>	SCHULZ J B ET AL: "Oxidative stress in patients with Friedreich ataxia." NEUROLOGY, vol. 55, no. 11, 12 December 2000 (2000-12-12), pages 1719-1721, XP001095770 ISSN: 0028-3878 abstract	21,22
Α	US 5 321 138 A (SPECTOR ABRAHAM ET AL) 14 June 1994 (1994-06-14) the whole document	1-26
Y	PALAU F: "FRIEDREICH'S ATAXIA AND FRATAXIN: MOLECULAR GENETICS, EVOLUATION AND PATHOGENESIS (REVIEW)" INTERNATIONAL JOURNAL OF MOLECULAR MEDICINE, SPANDIDOS, ATHENS, GR, vol. 7, no. 6, 2001, pages 581-589, XP001095758 ISSN: 1107-3756	21,22
A	the whole document	1-26

# INT NATIONAL SEARCH REPORT

Internation Application No
PCT/EP 03/07006

		PCT/EP 03/07006
C.(Continu	ation) DOCUMENTS CONSIDERED TO BE RELEVANT	
Category *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
Category * P, X		Relevant to claim No.  1–26



# INT NATIONAL SEARCH REPORT

Information on patent family members

Interna Pal Application No
PCT/EP 03/07006

Patent document cited in search report		Publication date	Patent family member(s)		Publication date
US 6133322	Α	17-10-2000	NONE		
US 5321138	Α	14-06-1994	US AU WO	5128365 A 6061990 A 9101125 A2	07-07-1992 22-02-1991 07-02-1991